NDA Whitepaper

Understanding Why the Patient Holds the Key to Successful Drug Development

Written by:
Dr Lisa Campbell, Senior Consultant & Medical Advisor at NDA Group

NDA
An SSI Strategy Company

About the author

Dr Lisa Campbell

Lisa is an experienced Medical Advisor with expertise in the scientific and regulatory aspects of clinical drug development in the EU and UK. She has a broad understanding of various therapy areas and trial phases, focusing on first-in-human trials, rare diseases, and innovative trial designs. Her interests include rare diseases, pediatrics, novel trial designs, patient engagement strategies, and the use of Patient Reported Outcomes.

Before joining NDA, Lisa spent nearly a decade at the MHRA, where she held leadership roles. She played a key role in developing a template protocol for platform trials during the pandemic and co-chaired the ICMRA 'Public Health Emergency Clinical Trials Working Group.'

Lisa's diverse background includes 12 years of practice as a medical doctor in Obstetrics and Gynaecology, with a specialty in Diabetes in Pregnancy. Lisa is committed to ensuring that the patient's voice is heard, and passionate about promoting inclusivity and diversity in clinical drug development.

The pharmaceutical industry is highly competitive, with numerous new drugs entering the market every year. For a drug to truly succeed, it needs to demonstrate clear advantages over existing therapies. Regulators and patients want to know what sets a new treatment apart and improves outcomes that matter most to those who are living with the disease. In many cases, it is the patient voice that illuminates these important differences and priorities.

Incorporating patient perspectives systematically throughout the drug development process has become a global priority that can define what makes or breaks a clinical development program. This approach, known as Patient-Focused Drug Development (PFDD), stands to reform how innovative solutions are identified, demonstrated, and approved to address unmet medical needs.

The Unique Patient Perspective

Patients living with a disease have direct, first-hand experience of its impact on their daily life, how they function both physically and emotionally, and what they consider meaningful change. For many complex, chronic conditions with heterogeneous manifestations, patients can offer a level of contextualised understanding that clinical experts simply cannot replicate. Their input provides a lens into how the illness affects sleep, work, relationships, independence, and overall quality of life. Patients also experience treatment burdens, side effects, risks, and limitations in a deeply personal way shaped by individual circumstances.

Currently, patients might provide input through informal consultations, formal scientific advice meetings or advisory boards, but their contribution is not systematically or uniformly incorporated across organisations and regions. Qualitative insights are not routinely quantitatively operationalised to inform decision making. New technologies now enable more convenient, widespread collection of patients' views. However, without agreed guidance on methodologies, this potentially widens variability rather than facilitating shared learnings. A standardized yet flexible framework for incorporating the patient voice throughout development could optimise efforts to address patient-defined unmet needs and align global clinical development programs.

How Patient Input Can Strengthen Development

Engaging patients systematically throughout the entire drug development cycle, from early research to post-market studies, is key to accomplishing the goals of PFDD. Initially, qualitative feedback from patients on disease impacts, experiences with current therapies, and desired treatment attributes can help guide early research priorities. Patient input then has the potential to be operationalised into outcome measures assessed in clinical trials to demonstrate impacts on aspects of disease and quality of life that patients identify as most meaningful.

Patient input may also have a part to play in decisions around whether or not payers endorse reimbursements and ultimately, therefore, whether the drug successfully ends up reaching patients. In 2019, simultaneous advice from EMA and HTA bodies was sought for the development of 27 medicines. Patients were actively involved in two-

thirds of these cases, emphasizing the growing recognition of the role patients play in shaping regulatory and reimbursement decisions. Designing clinical trials in partnership with patients can maximise trial participant retention rate and adherence to trial related activities. In addition, continued engagement with patients involved in trials also ensures that their perspectives and priorities are updated over time based on new data and understandings of safety and efficacy profiles emerging from studies. Finally, integrating quantitative data collected directly from patients on benefits and risks can strengthen regulatory applications by allowing regulators to accurately evaluate evidence supporting approval through a patient lens.

Proposed Best Practices & Global Guideline Needs

Several organizations have worked to establish best practices and guidance for PFDD. The FDA has published a series of PFDD guidance documents stemming from public workshops with patient groups [1]. These guidelines aim to provide methodological recommendations for systematically collecting and incorporating patient input throughout development and regulatory review.

"Incorporating patient perspectives systematically thoughout the drug development process has become a global priority that can define what makes or breaks a clinical development program."

Additionally, the International Council for Harmonisation (ICH) developed its first PFDD guidelines in 2019 to promote harmonized practices globally [2]. This harmonization aims to optimize efforts to address patient-defined unmet medical needs through more efficient and relevant drug development.

These guidance documents provide a foundation for systematically gathering and utilizing patient input to enhance medical product development and regulatory decision-making. Continued progress in this evolving area will depend on collaborative, multi-stakeholder efforts across several domains as listed below:

- **Guidance Refinement:** Feedback and real-world experiences systematically incorporating the patient perspective according to the methodological recommendations in PFDD guidance documents will help further refine and strengthen these best practices over time. Additional guidance may also aid implementation of topics like qualitative research methods.

- **Methodological Advancement:** Exploration of novel qualitative and quantitative techniques, as well as assessing existing methods, will strengthen the evidence base for patient-focused practices. Harmonization of standards globally also supports progress.

- **Expertise Development:** Ensuring training opportunities and expertise in patient engagement methodology for stakeholders involved in research and decision-making is important. Subject matter knowledge similarly supports quality implementation.

- **Resource Leveraging:** Complementary initiatives outside formal research, like in healthcare systems or using diverse data sources, offer opportunities to deepen understanding of patient needs. Learnings across settings can provide valuable insights.

An Opportunity for Impact

If the key stakeholders partner to establish internationally recognised best practices, the systematic integration of patient perspectives could transform medical product development. Both patients and industry stand to benefit from this shift - with potentially accelerated innovation, more efficient clinical trials that recruit better due to improved relevance, and consistent evidentiary standards worldwide to bring meaningful therapies faster to diverse patient populations globally.

Most importantly, by directly involving patients as collaborators, the research community has an opportunity to develop solutions addressing disease impacts that patients unanimously agree merit attention Granting patients ownership of their care also holds potential to drive better individual health outcomes through enhanced participant retention and compliance in clinical research. Such a movement may at long last generate therapies considered truly meaningful by those doing battle with illness each day.

Conclusion

Patient-Focused Drug Development offers a chance to build a future where patients' lived experiences, priorities, and partnership shape biomedical innovation from beginning to end. Realizing this vision depends on global cooperation between all key stakeholders to systematically operationalize and learn from the unmatched patient perspective. With harmonized international guidance on methodologies, the patient voice can be scaled to transform medical product development and deliver the transforma-tive change patients deserve worldwide. Now is the moment for action. Our expertise can provide you with; educational training in PFDD (touching upon patient experience data and clinical outcome as-sessments), bespoke recommendations for PFDD activities in your clinical program to support licensing and ultimately reimbursement and development of a patient engagement roadmap to help you navi-gate the wealth of PFDD opportunities globally.

Contact NDA at contact-us@ndareg.com to support you with your Patient Focused Drug Development Strategy.

We bring men, women and children, all over the world, the best possible medicines without unnecessary delay

Contact us to learn how our experts can support you and your approval

NDA An SSI Strategy Company

www.ndareg.com